

Identification and isolation of transplantable human hematopoietic stem cells from pluripotent cell lines; two steps from primitive hematopoiesis to transplantable definitive cells, and non-toxic conditioning of hosts for hematopoietic stem cell transp...

Grant Award Details

Identification and isolation of transplantable human hematopoietic stem cells from pluripotent cell lines; two steps from primitive hematopoiesis to transplantable definitive cells, and non-toxic conditioning of hosts for hematopoietic stem cell transplants

Grant Type: Tools and Technologies III

Grant Number: RT3-07683

Project Objective: To address two bottlenecks to developing hESC-derived HSCs for transplant:

inability to generate engraftable, "definitive" HSCs from hESC due to lack of fundamental knowledge of their biology and
lack of safe conditioning regimens for enabling HSC transplant

Investigator:

Name: Irving Weissman
Institution: Stanford University
Type: PI

Name: Judith Shizuru
Institution: Stanford University
Type: Co-PI

Disease Focus: Blood Disorders, Blood Cancer, Cancer

Human Stem Cell Use: Adult Stem Cell

Award Value: \$1,271,952

Status: Active

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Identification and isolation of transplantable human hematopoietic stem cells from pluripotent cell lines; two steps from primitive hematopoiesis to transplantable definitive cells, and non-toxic conditioning of hosts for hematopoietic stem cell transp...

Public Abstract: A goal of stem-cell therapy is to transplant into a patient "tissue-specific" stem cells, which can regenerate a particular type of healthy tissue (e.g., heart or blood cells). A major obstacle to this goal is obtaining tissue-specific stem cells that (1) are available in sufficient numbers; and (2) will not be rejected by the recipient. One approach to these challenges is to generate tissue-specific stem cells in the lab from "pluripotent" stem cells, which can produce all types of tissue-specific stem cells. The rationale is that pluripotent stem cells that will be tolerated are easier to directly obtain than tissue-specific stem cells that will be tolerated. Furthermore, descendants of a tolerated pluripotent stem cell will also be tolerated and can be produced abundantly.

The goal of the proposed project is to develop techniques for generating transplantable blood-forming stem cells from pluripotent stem cells. In pursuit of this goal, we will study how blood-forming stem cells arise during development. We will also test new methods--less toxic than current chemotherapy and radiation--for preparing recipients for transplantation of blood-forming stem cells.

Additional benefit: Successful transplantation of blood-forming stem cells allows the recipient to tolerate other tissue or organ transplants from the same donor. Thus, transplanted blood-forming stem cells could allow people to receive organs that they may otherwise reject, without taking immune-suppressing drugs.

Statement of Benefit to California: We aim to generate from stem cells that can produce all tissues of the body those stem cells that specifically form blood. We will also test new methods--less toxic than current chemotherapy and radiation--for pretreatment before transplantation of blood-forming stem cells. A large number of patients in California could benefit from advances in this field, primarily those with diseases affecting the production of blood and immune cells: leukemia, lymphoma, thalassemia, certain types of anemia, immune deficiency diseases, autoimmune diseases (e.g., lupus), etc. For leukemia and lymphoma alone, in 2014 in California, there will be an estimated 12,060 newly diagnosed cases, 103,400 existing cases, and 4,620 deaths (per the California Cancer Registry). The cost of these blood cancers are difficult to estimate but they account for 6% of cancers in women and 9% in men in California, where the estimated cost of cancer per year is \$28.3 billion.

The reagents generated in these studies can be patented, forming an intellectual property portfolio shared by the state. The funds generated from the licensing of these technologies will provide revenue for the state, help increase hiring of faculty and staff (many of whom will bring in other, out-of-state funds to support their research) and could reduce the costs of related clinical trials. Only California businesses are likely to be able to license these reagents and to develop them into diagnostic and therapeutic entities.

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